

Fig. 1

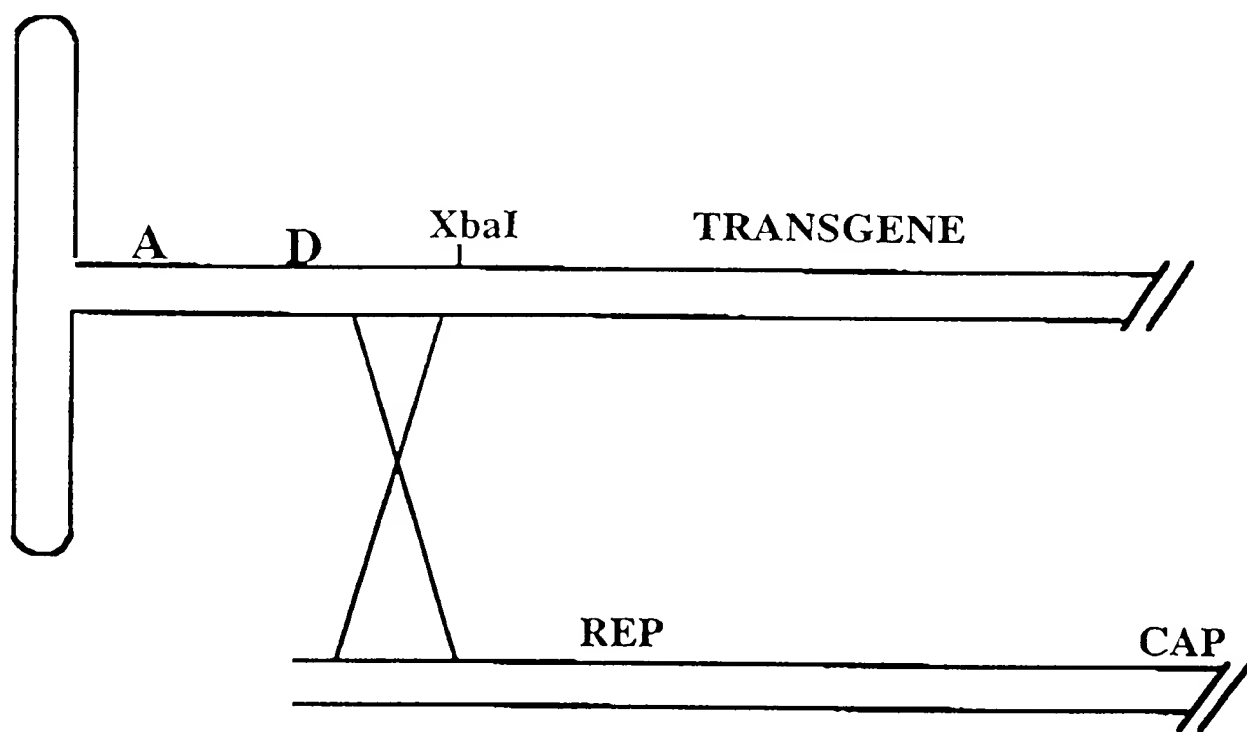
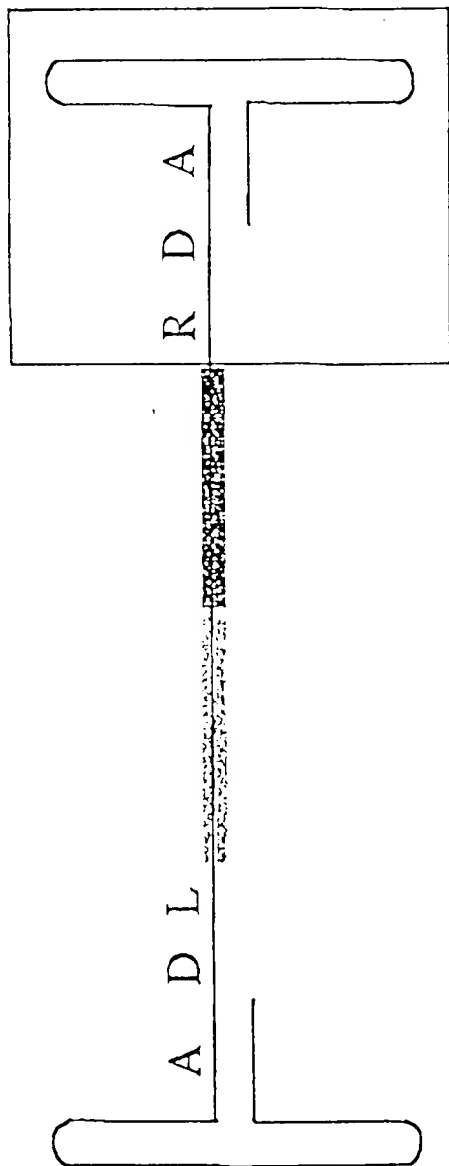


Fig. 2

wild-type



pSub201

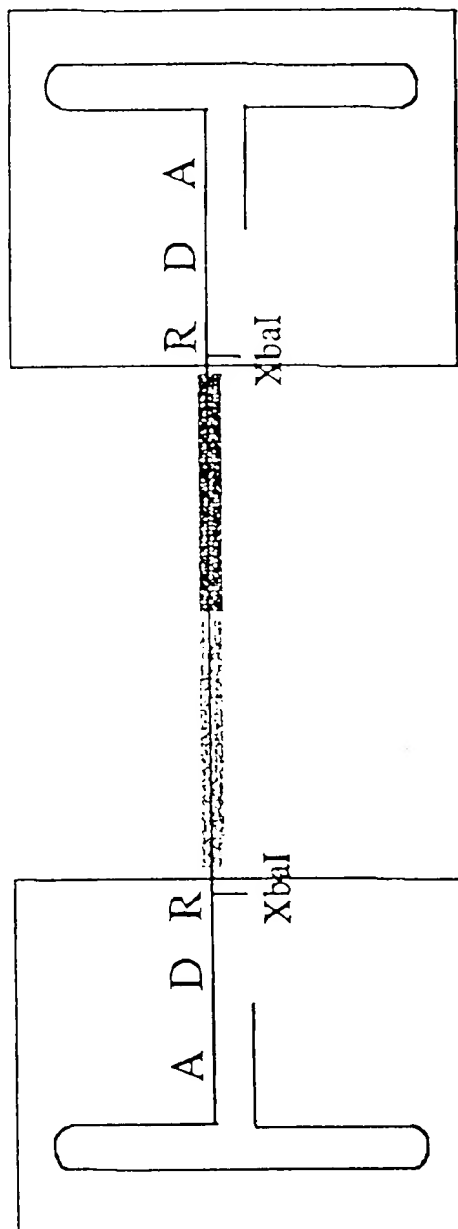


Fig. 3

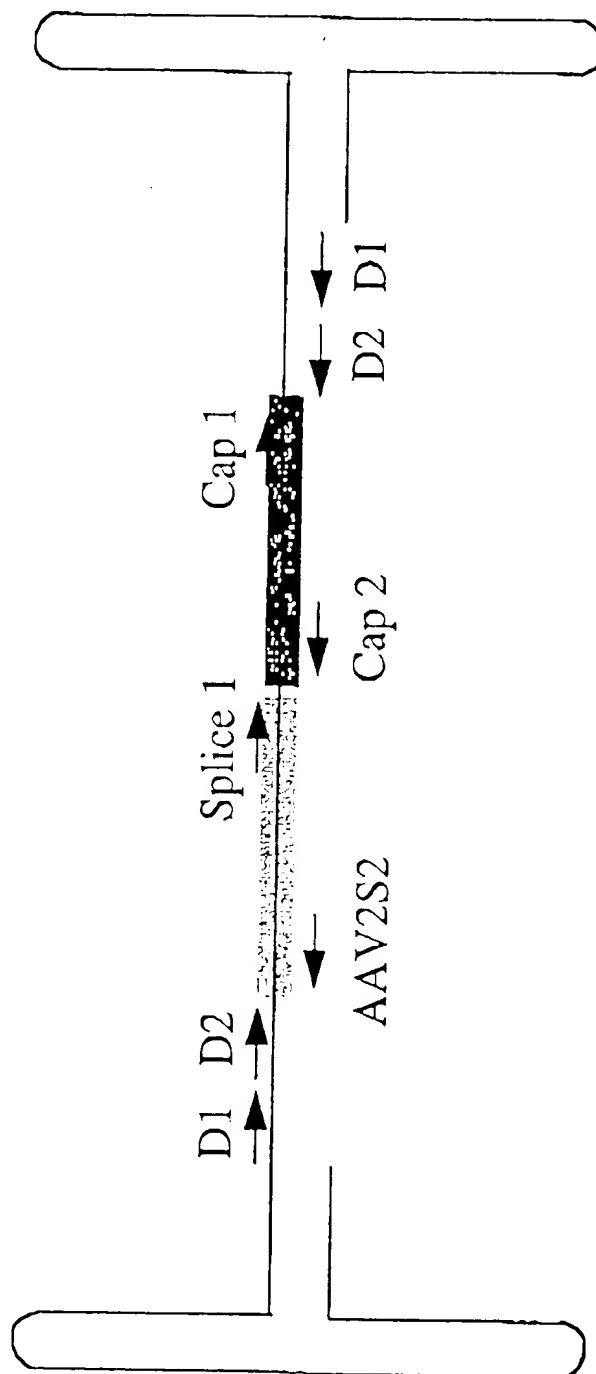


Fig. 4

Wild-Type

AGAGGGAGTGGCCAA CTCCATCACTAGGGGTTCCCT GGAGGGGTGGAGTCGTGACGTGAATTACGTCATAGGGTTAGGGAGGTCC

A                      D                      L

D1 oligo →

pSub201

AGAGGGAGTGGCCAA CTCCATCACTAGGGGTTCCCT TGTAGTTAATGATTAAACCCGCCCATGCTACTTATCTACGTAGCCATGCTCTAGA

A                      D                      R

D1 oligo → D2 oligo → XbaI

MEG → Human FIX → GH:PA

Fig. 6

# rAAV Production

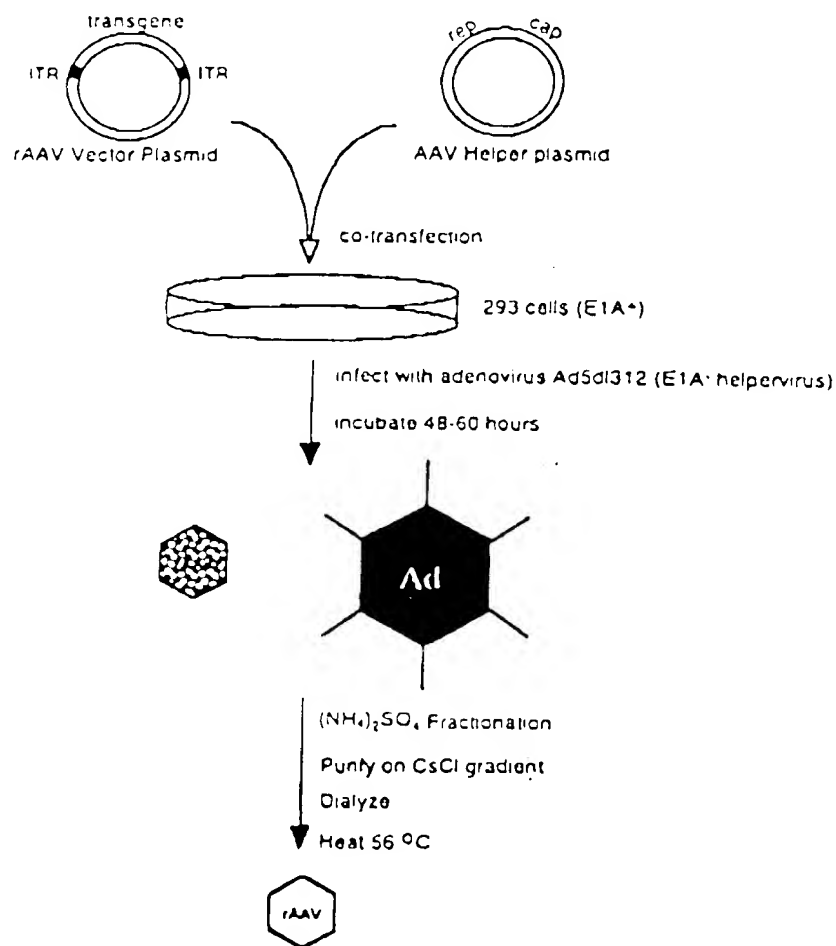


Fig. 7

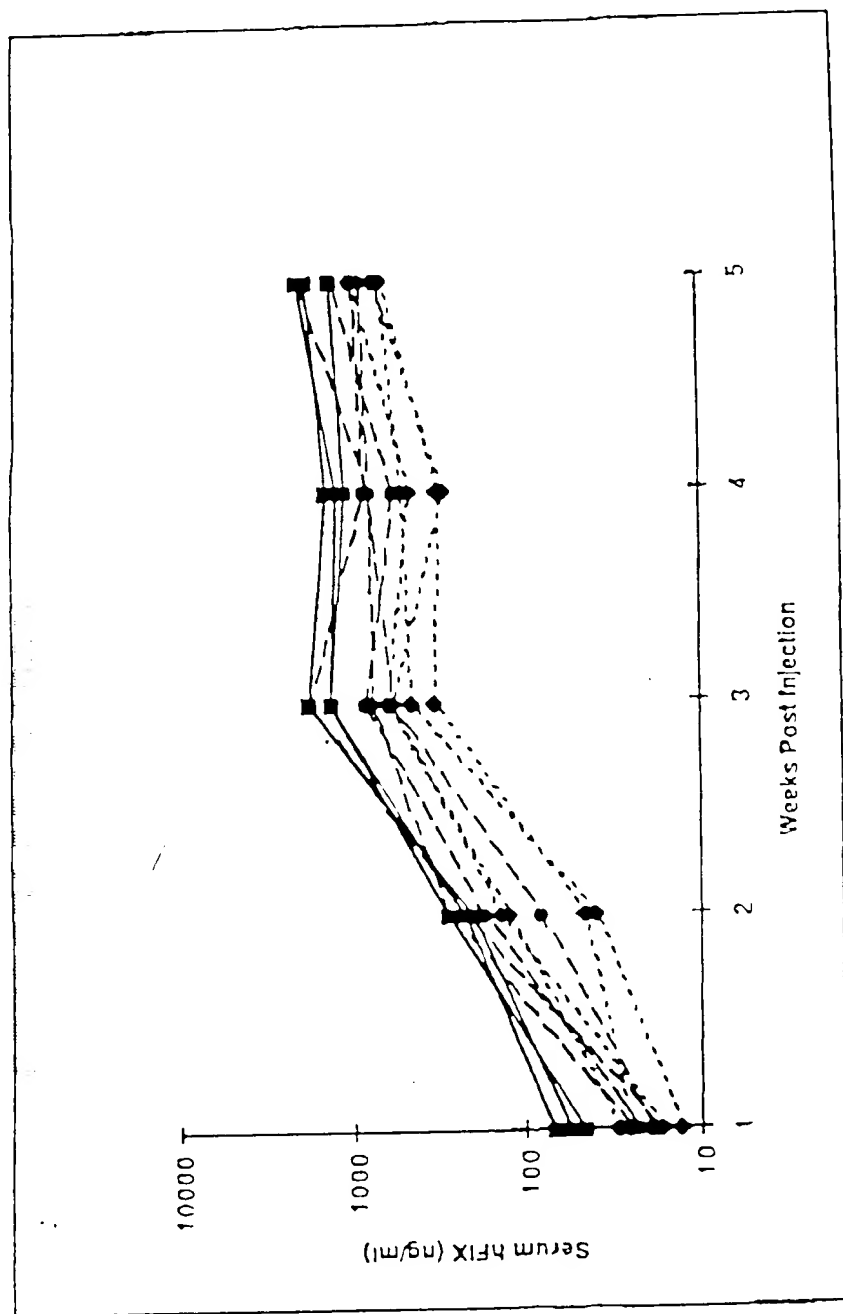
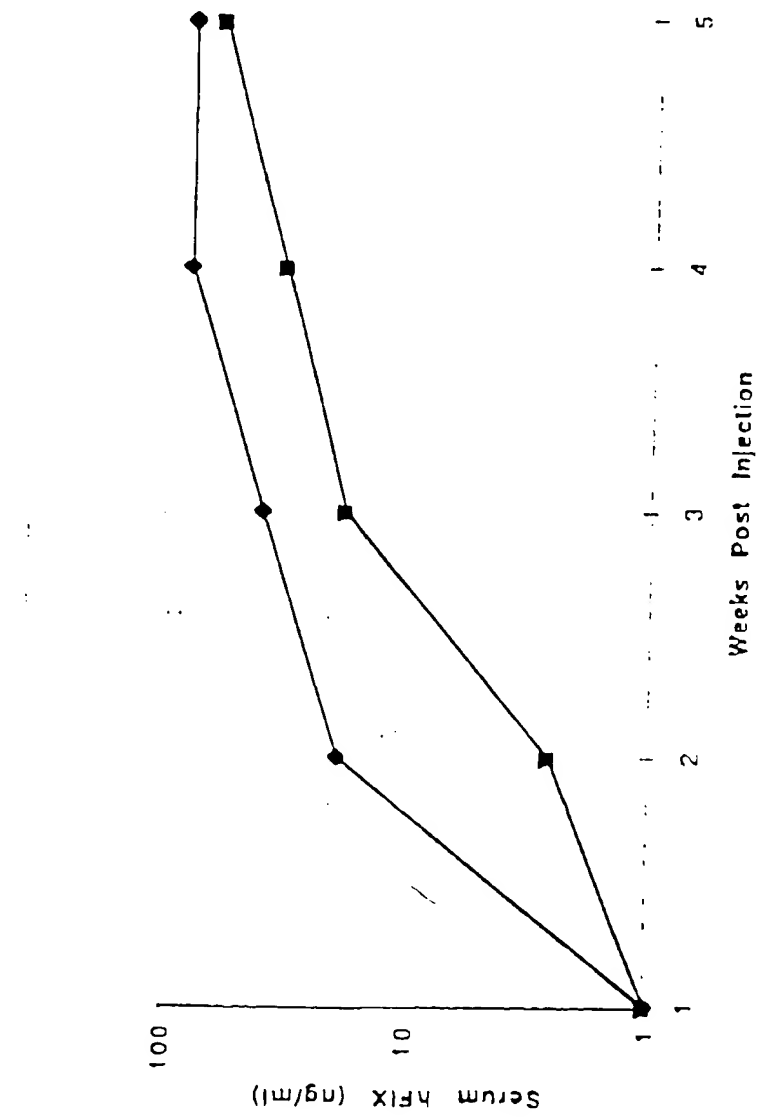


Fig. 8





— 4.2X10<sup>10</sup> Particles (n=2)

Fig. 9

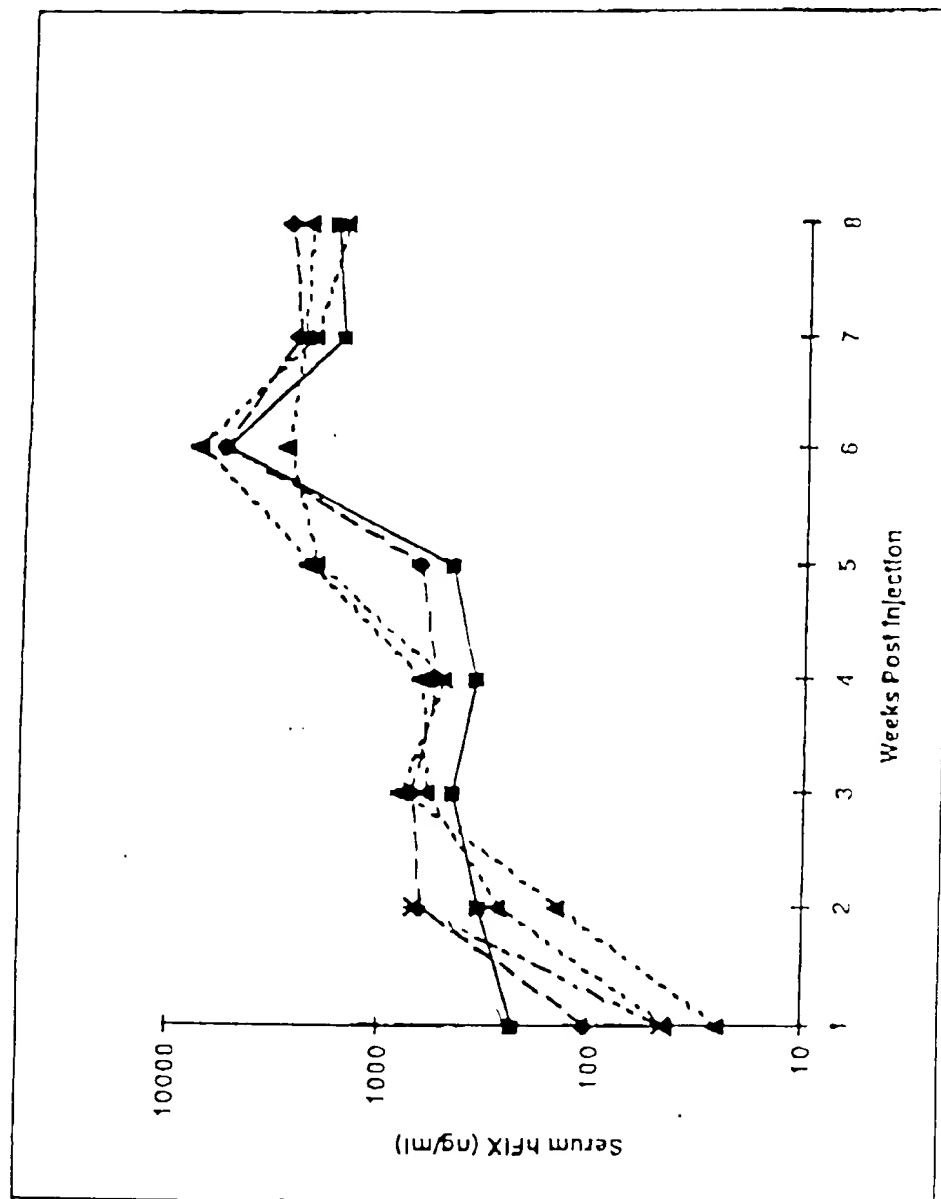


Fig. 10

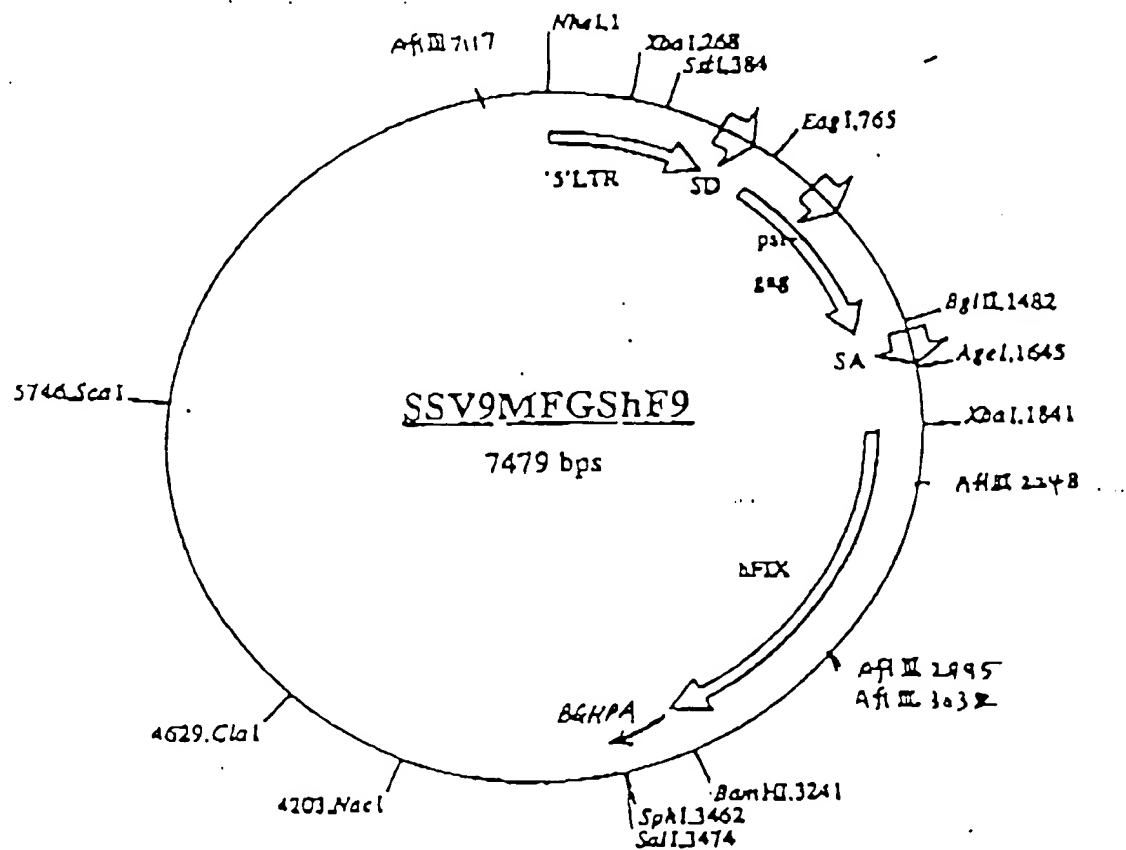


Fig. 11